

Challenge TDT

Allogeneic Haematopoietic Stem Cell Transplant (HSCT)

is a Treatment Option That Has the Potential to Correct the Genetic Deficiency in β -Thalassaemia^{1,2,3}

KEY POINTS

Allogeneic haematopoietic stem cell transplant (HSCT) is a treatment option with the potential to correct the genetic deficiency in TDT.^{1,2}

In a retrospective study of 1493 patients with thalassaemia major who underwent transplantation between 2000 and 2010, two-year overall survival and thalassaemia-free survival were estimated to be 88% and 81%, respectively.³

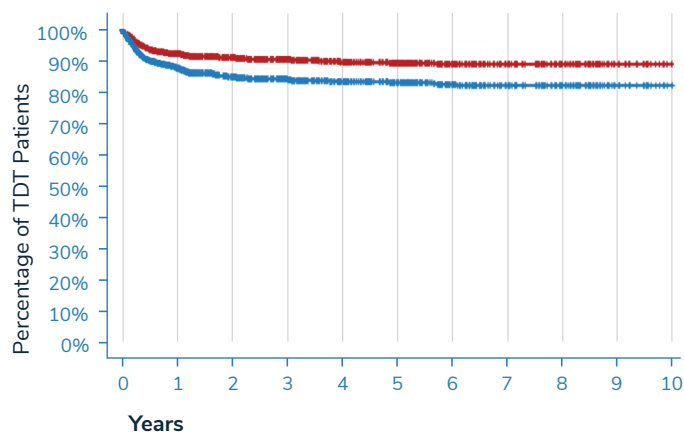
HSCT is generally performed in younger patients with HLA-matched donors.^{3,4,5}

Many patients with TDT do not receive allogeneic HSCT due to increased risk of mortality stemming from the lack of a suitable matched donor, presence of existing complications, and/or age.^{1,3,4,5}

REFERENCES:

- Guidelines for the Management of Transfusion Dependent Thalassaemia (TDT). 3rd ed. *Thalassaemia International Federation*. 2014. ISBN-13:978-9963-717-06-4.
- Lucarelli G, Isgrò A, Sodani P, Gaziev J. Hematopoietic stem cell transplantation in thalassaemia and sickle cell anemia. *Cold Spring Harb Perspect Med*. 2012. doi:10.1101/cshperspect.a011825.
- Baronciani D, Angelucci E, Potschger U, et al. Hematopoietic stem cell transplantation in thalassaemia: a report from the European Society for Blood and Bone Marrow Transplantation Hemoglobinopathy Registry 2000–2010. *Bone Marrow Transplant*. 2016;51(4):536-541.
- Angelucci E, Matthes-Martin S, Baronciani D, et al. Hematopoietic stem cell transplantation in thalassaemia major and sickle cell disease: indications and management recommendations from an international expert panel. *Haematologica*. 2014;99(5):811-820.
- Sabloff M, Chandu M, Wang Z, et al. HLA-matched sibling bone marrow transplantation for β -thalassaemia major. *Blood*. 2011;117(5):1745-1750.

Survival Rates for 1493 HSCT Recipients With Thalassaemia During the Period 2000-2010³



	Patients	Events	Probability
A. Overall Survival (2 years)	1493	154	0.88±0.01
B. Thalassaemia-Free Survival (2 years)	1493	253	0.81±0.01

Survival rates for 1493 transplant recipients in the period 2000-2010.

Overall Survival (A) and Thalassaemia-Free Survival (B) are shown.

Adapted from Baronciani D, Angelucci E, Potschger U, et al. Hematopoietic stem cell transplantation in thalassaemia: a report from the European Society for Blood and Bone Marrow Transplantation Hemoglobinopathy Registry, 2000–2010. *Bone Marrow Transplant*. 2016;51(4):536-541.